Precigen Completes Submission of BLA with Request for Priority Review to the FDA for PRGN-2012 for the Treatment of Adults with Recurrent Respiratory Papillomatosis

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– PRGN-2012 has the potential to be the first FDA-approved therapeutic for the treatment of adults with RRP, a rare and devastating chronic disease for which the current standard-of-care is repeated surgeries –

– PRGN-2012 received Breakthrough Therapy Designation from the FDA and Orphan Drug Designation from the FDA and the European Commission –

– The BLA, under an accelerated approval pathway, is supported by data from the Phase 1/2 pivotal study in which more than 50% of patients achieved Complete Response and more than 85% of patients had a decrease in surgical interventions in the year after PRGN-2012 treatment compared to the year prior to treatment –

– PRGN-2012 was well-tolerated with no dose-limiting toxicities and no treatment-related adverse events greater than Grade 2 –

GERMANTOWN, Md., Dec. 30, 2024 /PRNewswire/ -- Precigen, Inc. (Nasdaq: PGEN), a biopharmaceutical company specializing in the development of innovative gene and cell therapies to improve the lives of patients, today announced the completion of the rolling submission for a biologics license application (BLA) to the US Food and Drug Administration (FDA) for PRGN-2012 (INN: zopapogene imadenovec†) for the treatment of adult patients with recurrent respiratory papillomatosis (RRP). The submission is in the initial 60 day review period, during which time the FDA will decide whether to accept the BLA for further review and set the Prescription Drug User Fee Act (PDUFA) action date. The BLA included a request for priority review, which, if granted, would reduce the review timeline from the standard 10-month to a priority 6-month review from the date the submission is accepted by the FDA.

PRGN-2012 is an investigational AdenoVerse® gene therapy designed to elicit immune responses directed against cells infected with human papillomavirus (HPV) 6 or HPV 11. PRGN-2012 received Breakthrough Therapy Designation, Orphan Drug Designation, and an accelerated approval pathway from the US Food and Drug Administration (FDA), and Orphan Drug Designation from the European Commission.

PRGN-2012 has the potential to be the first FDA-approved therapeutic for the treatment of adults with RRP. RRP is a rare, difficult-to-treat, lifelong neoplastic disease of the upper and lower respiratory tracts caused by infection with HPV 6 or HPV 11 that can be fatal. Currently, there is no cure for RRP and the current standard-of-care is repeated surgeries, which do not address the underlying cause of disease and are associated with significant morbidity. As a result, the cycle of recurrences and surgeries continues and patients can require hundreds of lifetime surgeries.[1-7]

The BLA is supported by data from the pivotal Phase 1/2 clinical study of PRGN-2012 for the treatment of RRP (NCT04724980), which evaluated the safety and efficacy of PRGN-2012 in adult RRP patients. Of the 38 total patients enrolled in the study, three patients received four administrations of PRGN-2012 at 1x 10^{11} particle units (PU)/dose and 35 patients received four administrations of PRGN-2012 at the recommended phase 2 dose (RP2D) of 5 x 10^{11} PU/dose over a 12 week treatment period via subcutaneous injection. Primary endpoints included safety and Complete Response rate defined as the percentage of patients who require no RRP surgeries in the 12-month period after PRGN-2012 treatment completion. Key secondary endpoints included HPV-specific immune responses, extent of papilloma growth as measured by Derkay scoring, and quality of life as measured by Vocal Handicap Index-10 (VHI-10). As reported in the groundbreaking results from the pivotal study presented at the 2024 American Society of Clinical Oncology (ASCO) annual meeting, the primary safety and efficacy endpoints were met.

"The impact of this debilitating disease on patients, families, and their caregivers has been overlooked for more than half a century. There is currently no approved therapy for RRP patients and the submission of our BLA is an extremely important step in bringing the first therapy to fight this devastating disease," said Helen Sabzevari, PhD, President and CEO of Precigen. "We look forward to working closely with the FDA on next steps now that we have completed the BLA submission and we are excited by the potential to bring PRGN-2012 to RRP patients as quickly as possible. With our most recent financial transactions announced last week to enhance our balance sheet, we have extended our cash runway into 2026, well beyond

potential commercial launch in the second half of 2025."

AdenoVerse®
Precigen's AdenoVerse platform utilizes a library of proprietary adenovectors for the efficient gene delivery of therapeutic effectors, immunomodulators, and vaccine antigens designed to modulate the immune system. Precigen's gorilla adenovectors, part of the AdenoVerse library, have potentially superior performance characteristics as compared to current competition. AdenoVerse gene therapies have been shown to generate high-level and durable antigen-specific T-cell immune responses as well as an ability to boost these responses via repeat administration. Superior performance characteristics and high yield manufacturing of AdenoVerse vectors leveraging UltraVector® technology allows Precigen to engineer cutting-edge investigational gene therapies to treat complex diseases.

Precigen: Advancing Medicine with Precision ™
Precigen (Nasdaq: PGEN) is a dedicated discovery and clinical stage biopharmaceutical company advancing the next generation of gene and cell therapies using precision technology to target the most urgent and intractable diseases in our core therapeutic areas of immuno-oncology, autoimmune disorders, and infectious diseases. Our technologies enable us to find innovative solutions for affordable biotherapeutics in a controlled manner. Precigen operates as an innovation engine progressing a preclinical and clinical pipeline of well-differentiated therapies toward clinical proof-of-concept and commercialization. For more information about Precigen, visit www.precigen.com or follow us on LinkedIn or YouTube.

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Cautionary Statement Regarding Forward-Looking Statements
Some of the statements made in this press release are forward-looking statements. These forward-looking statements are based upon the Company's current expectations and projections about future events and generally relate to plans, objectives, and expectations for the development of the Company's business, including the timing and progress of preclinical studies, clinical trials, regulatory approvals, commercial launches and related milestones, the promise of the Company's portfolio of therapies, and in particular its CAR-T and AdenoVerse therapies. Although management believes that the plans and objectives reflected in or suggested by these forward-looking statements are reasonable, all forward-looking statements involve risks and uncertainties and actual future results may be materially different from the plans, objectives and expectations expressed in this press release. The Company has no obligation to provide any updates to these forward-looking statements even if its expectations change. All forward-looking statements are expressly qualified in their entirety by this cautionary statement. For further information on potential risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ from those contained in the forward-looking statements, see the section entitled "Risk Factors" in the Company's most recent Annual Report on Form 10-K and subsequent reports filed with the Securities and Exchange Commission.

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†zopapogene imadenovec is the international nonproprietary name (INN) for the investigational therapeutic known as PRGN-2012. Zopapogene imadenovec has not been approved by any health authority in any country for any indication.

References

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